Mallinckrodt Pharmaceuticals

November 6, 2018

Dear Joslyn:

As a follow-up to our letter of October 1st, and as a valued advocacy partner, we wanted to make you aware that this week, Mallinckrodt communicated the topline findings of our recently completed registration trial for VTS-270, our development product to treat Niemann-Pick Type C, a complicated, ultra-rare neurodegenerative disease that typically presents in childhood and is ultimately fatal.

In the trial, the product did not show a statistically significant separation from placebo, but importantly, neither the VTS-270, nor the placebo arm showed disease progression—as would have been anticipated in a neurodegenerative condition over 52 weeks of observation. The expectation was that both treatment groups in the study would worsen over the trial period but that the VTS-270 treated group might show an attenuated course based on an accrued benefit.

Accordingly, our review of the data from the Phase 2b/3 trial, has required substantial effort and still continues. We expect a better understanding of the potential benefit of VTS-270 to emerge as we carefully consider the totality of data available to us. This is an important step. At a meeting in August the FDA indicated to us that their view on the potential approvability for VTS-270 will be based on the totality of data, not a single study or endpoint and in the coming months we will continue to work with the primary investigators and the FDA to clarify a potential path forward.

Current studies and expanded access/compassionate use programs currently in place will continue. We understand the importance of pursuing this potential treatment of Niemann-Pick Type C, and based on our current assessment of the safety data, at this time we believe that continued treatment with VTS-270 in the ongoing open label portion of the trial is acceptable. Patients, their families, and our patient group partners, should know that we remain deeply committed to this work. Please do not hesitate to contact us with questions about this matter.

Sincerely,

Sheila Talafous
Director, Advocacy Relations

Derek Naten
Senior Director, Government Relations & Advocacy